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A new phenotypic screening platform that identifies biologically-relevant targets and lead compounds for the treatment of Parkinson's disease

### Grant Award Details

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A new phenotypic screening platform that identifies biologically-relevant targets and lead compounds for the treatment of Parkinson's disease

**Grant Type:** Inception - Discovery Stage Research Projects

**Grant Number:** DISC1-10674

**Investigator:**

<b>Name:</b>	Vicki Nienaber
<b>Institution:</b>	Zenobia Therapeutics
<b>Type:</b>	PI

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**Disease Focus:** Parkinson's Disease, Neurological Disorders

**Award Value:** \$150,000

**Status:** Pre-Active

### Grant Application Details

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**Application Title:** A new phenotypic screening platform that identifies biologically-relevant targets and lead compounds for the treatment of Parkinson's disease

**Public Abstract:****Research Objective**

Demonstrate that our HitFinder™ library can be screened for phenotypic changes in A53T-IPSC-derived dopaminergic neurons and use a secondary handle to identify the targets responsible.

**Impact**

This technology combines phenotypic screening and target-ID eliminating the need to bias assays and/or screening libraries permitting application directly in iPSC-derived cells.

**Major Proposed Activities**

- Prepare screening library including purchase of compounds and addition of chemical handles for target identification
- Screen library for phenotypic changes in iPSC-derived engineered A53T-synuclein dopaminergic neurons: single point followed by dose-response
- Large-scale preparation of compound-target complex in A53T IPSC-DA-neurons under conditions of phenotypic assay and confirm phenotypic change for target-ID.
- Process scaled-up A53T-DA neurons and attach an affinity tag to the compound-target complex. Identify number of targets that reacted with the ligand (selectivity) and the identity of these targets.

**Statement of Benefit to California:**

This technology has the potential for broad impact on patients. Immediately, compounds and targets identified from this screen can progress into a drug discovery program to identify new treatments for Parkinson's disease (PD). PD is estimated to affect 36-60,000 Californians. Application of iPSC-derived neurons permits screening in patient-derived cells to determine if therapeutics/targets are relevant in all forms of PD (genetic and sporadic) and eventually expand to other diseases.

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